Research to cure fatal childhood disease, combat Zika and slow Parkinson's get go-ahead from Stem Cell Agency Board

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Oakland, CA – Finding a new way to treat a deadly or disabling condition takes imagination, a lot of hard work and money. Today the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded more than $20.5 million to 11 projects whose goal is to identify and explore promising new stem cell therapies or technologies to improve patient care.

The award is part of CIRM's Quest program which promotes the discovery of stem cell-based approaches, creating a pipeline of projects that will be ready to move to the next stage of development within two years.

"At CIRM we never underestimate the importance of early stage scientific research; it is the birth place of groundbreaking discoveries," says C. Randal Mills, Ph.D., President and CEO of CIRM. "We hope these Quest awards will not only help these incredibly creative researchers deepen our understanding of several different diseases, but also lead to new approaches on how best to use stem cells to develop treatments."

Among the successful applicants is Stanford's Rosa Bacchetta, who plans on using a gene editing technique to repair a patient's own blood stem cells to cure IPEX, a rare but fatal childhood disease.

In a letter to the CIRM Board Bacchetta says this could potentially benefit people with other similar diseases. "Although it is a rare disease, IPEX is a prototype of a series of diseases with autoimmunity of genetic origin that overall severely affect children at a very early age. This expertise has given us a unique path forward in developing a definitive cure for this devastating genetic disease."

U.C. San Diego's Alysson Muotri wants to study how the Zika virus affects the human brain. In previous studied he identified an anti-viral medication that has already been approved by the Food and Drug Administration (FDA) for a different condition. He now wants to see if that medication can block the virus from spreading and in turn help treat infected individuals.

Birgitt Schuele of The Parkinson's Institute wants to use CRISPR/dCas9, a powerful new gene editing tool, to lower levels of a protein in the brain that has been linked to Parkinson's disease, hopefully slowing down the progression of the disease in people.

Here is the list of successful applicants.

Application	Title	Institution	ICOC Committed funding
DISC2-09526	Gene editing for FOXP3 in human HSC	R. Bacchetta – Stanford University	$1,100,568
DISC2-09649	Treatment for Zika virus infection and neuroprotection efficacy	A. Muotri – U.C. San Diego	$2,117,880
DISC2-09565	Preclinical development of human hepatocyte progenitor cells for cell therapy	R. Nusse – Stanford University	$1,655,436

DISC2-09615	Targeted off-the-shelf immunotherapy to treat refractory cancers	D. Kaufman – U.C. San Diego	$2,134,868
DISC2-09569	hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington's Disease	L. Thompson – U.C. Irvine	$1,787,543
DISC2-09624	Protein tyrosine phosphatase – sigma inhibitors for hematopoietic regeneration	J. Chute – U.C. Los Angeles	$2,116,708
DISC2-09596	Direct cardiac reprogramming for regenerative medicine	D. Srivastava – Gladstone Institutes	$2,400,048
DISC2-09635	Designing a cellular niche for transplantation of human embryonic stem cell-derived beta cells	J. Sneddon – U.C. San Francisco	$2,006,076
DISC2-09559	Thin film encapsulation devices for human stem cell derived insulin producing cells	T. Desai – U.C. San Francisco	$1,092,063
DISC2-09610	CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease	B. Schuele – Parkinson's Institute	$1,931,589
DISC2-09637	Genome editing to correct cystic fibrosis mutations in airway stem cells	M. Porteus – Stanford University	$2,201,136

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov

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